Statement of Intent of the Rare Diseases Working Group Relating to Patient Access to Rare Disease Treatment in South Africa

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1. **Current Context:**
   - South Africa has two distinct healthcare sectors, the medicals schemes sector which accounts for 8.8 million lives and the public sector which caters for the majority of South Africa patient's healthcare needs.
   - South Africa is currently undergoing a healthcare reform with NHI scheduled to take effect from 2026.
   - For many rare diseases, basic knowledge such as the cause of the disease, pathophysiology, natural course of the disease and epidemiological data is limited or not available. This significantly hampers the ability to diagnose and treat these diseases. To address this challenge, public funding of fundamental research into the disease process remains necessary both at the national and global level.[1]
   - There is currently no legislation nor national plan for rare diseases in South Africa
   - Specifically, with reference to managing rare diseases in South Africa, we are faced with the following resource constraints:
     - Not many centres of excellences exist.
     - Rare disease patients are scattered across countries. As a result, medical expertise for each of these diseases is a scarce resource. Doctors and nurses need to be adequately trained to treat rare diseases (especially in rural areas).
     - We have a lack of epidemiological data available and registries will need to be established.
     - Funding constraints
   - **Patients with Rare Diseases**[2] today have -variable access and reimbursement predictability or certainty within the medical scheme or the public sector due to the lack of a national Rare Disease policy, and the ambiguity created by the current PMB framework which governs minimum levels of care in the medical schemes sector.
   - **Pharmaceutical Companies** have no planning predictability or certainty in terms of will their molecules be required by Rare Disease patients in the South African market due to lack of Rare Disease patient access and reimbursement policy and guidelines.

2. **The need and express intent of a joint medical schemes, medical scheme administrators, managed care companies, pharmaceutical industry, medical technology industry and patient association working group to address patient access to rare disease:**

   - Medical schemes, medical scheme administrators, managed care companies, pharmaceutical industry, medical technology industry and rare disease patient associations wish to form a collaborative working group with the express intention to:

[1] https://www.who.int/medicines/areas/priority_medicines/Ch6_19Rare.pdf

[2] Non common definition exists in South Africa for Rare Diseases. This will need to be developed. In Europe, a disease is considered to be rare when it affects 1 person per 2000 lives.

- Address the current variability of rare disease patients access and reimbursement predictability and certainty.
- To work towards addressing the lack of predictability and certainty for all stakeholders, by developing guiding principles and framework for rare diseases.
- Work in a stakeholder inclusive process basis to improve rare disease patient access and treatment with innovative solutions in a sustainable manner.
- Consider international best practice in developing a unique South African document with guiding principles and framework for rare diseases. Find and build consensus among the working group and key stakeholders on an appropriate, sustainable rare diseases framework for South African rare disease patients.